The results of the primary analysis at 24 weeks have been previously reported. Two of 56 patients discontinued treatment during Period 2 (1 adverse event [AE], 1 consent withdrawn). The study met its primary endpoint; both doses of vamorolone showed statistically significant and clinically meaningful improvement in functional outcomes in patients who received either vamorolone 2 mg/kg/day or vamorolone 6 mg/kg/day in Period 1 and continued on vamorolone in Period 2.

### Summary

- Baseline characteristics were similar across groups for age and most functional outcome (Table 1).

- In TTSTAND velocity, the effect size of Week 48 vs Week 24 was maintained until Week 48, with a significant difference vs. vamorolone 2 mg/kg/day at Week 48 (p < 0.001) (Figure 2).

- In TTCLIMB velocity, the effect size of Week 24 vs Week 0 was maintained until Week 48, with a significant difference vs. vamorolone 2 mg/kg/day at Week 48 (p < 0.001) (Figure 2).

- In TTRW velocity, differences between dose groups were maintained until Week 48 in mITT-2 and TTRW-2 (p < 0.01), while it was not in TTRW (p > 0.05) or TTRW-2 (p > 0.05) (Figure 2).

- Three serious AEs were reported during the 48 weeks: perforated appendixes in 2 patients, retinopathy in 1 patient, and a Guillain-Barré syndrome 2 weeks after the last dose in one patient.

- The most common AEs reported during the 48-week treatment were: Upper respiratory tract infections (vamorolone 2 mg/kg/day: 7.7% vs 11.7% vamorolone 6 mg/kg/day: 14.3% vs placebo); Vomiting (vamorolone 2 mg/kg/day: 21.4% vs 6 mg/kg/day: 14.4% vs placebo); Constipation/fecal incontinence (vamorolone 2 mg/kg/day: 12.5% vs 6 mg/kg/day: 10.7% vs placebo); Amelioration therapy (vamorolone 2 mg/kg/day: 25.7% vs 17.3% placebo); Diarrhoea (vamorolone 2 mg/kg/day: 3.5% vs placebo); Fatty liver (vamorolone 2 mg/kg/day: 6% vs placebo: 17.5% vs placebo).

- There were no increases in plasma levels of adverse events from Period 1 to Period 2 for any of the vamorolone doses (Figure 6).

- No starting of growth was observed with either vamorolone dose.

- The growth velocity height indicated a height rate that is greater than the increase in height rate in the average age-matched population in keeping with known natural history (Table 1).

### Conclusions

- Efficacy of vamorolone 6 mg/kg/day established at 24 weeks was maintained over 48 weeks across all outcome measures, while only across some measures for 2 mg/kg/day.

- Vamorolone treatment was generally well tolerated at both dose levels throughout 48 weeks.

- For subjects who continued on the same dose of vamorolone throughout the 48 weeks, the proportion of patients achieving some measures for 2 mg/kg/day.

- The longer-term results of the VISION-DMD (VBP15-004) study confirm safety and efficacy of vamorolone with a different safety profile and no starting of growth.

### References

3. Hoffman EP. Presented at PPMD Annual Conference, June 22–26, 2021 (Virtual)

### Acknowledgments

We would like to thank all patients and their families for sharing their experiences with us.

### Abbreviations

- BMI: Body mass index
- BMD: Bone mineral density
- DMD: Duchenne muscular dystrophy
- SD: Standard deviation
- SEM: Standard error of the mean
- TTCLIMB: Time to climb 4 steps
- TTRW: Time to run/walk
- VAM: Vamorolone
- VICTA: Vichai and Tanarat Aothasathan Foundation
- VISION-DMD: Vamorolone in Duchenne Muscular Dystrophy (VBP15-004) study
- mITT: Modified intention to treat
- mITT-2: Modified intention to treat-2 population
- TTRW: Time to run/walk
- TTSTAND: Time to stand from supine
- VAM: Vamorolone
- VAM 2 mg/kg (n=26)
- VAM 6 mg/kg (n=30)
- VAM 2 mg/kg/day (n=28)
- VAM 6 mg/kg/day (n=28)
- VAM 6 mg/kg/day vamorolone (n=28)
- VAM 6 mg/kg/day placebo (n=28)
- VAM 2 mg/kg/day placebo (n=28)
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